New 'How To' Guide Offers Companies Help on Moving Stem Cell Therapies to Patients

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San Francisco, CA – Moving promising therapies from the lab into clinical trials is one of the biggest challenges in developing new drugs. This is doubly true with stem cell and other cell-based therapies. Now a new tool is available to guide researchers, companies, and product developers through the process and increase their chances of success.

The tool is an article published in the journal Stem Cells Translational Medicine entitled "Communications on the Development Pathway for a Cell-Based Therapy: Why, What, When, and How?" It lays out the process involved in working with the US Food and Drug Administration (FDA) and details the different opportunities that companies have to work with the FDA to clear the path to clinical trials.

"The field of regenerative medicine is still relatively young and involves new and novel science," says Ellen Feigal, MD, Senior Vice President for Research and Development at the California Institute for Regenerative Medicine (CIRM), and the lead author of the article. "The products involved might be classified as a biologic - such as human cells and tissues or gene therapy- a device, a drug or combination of those. They also span a range of areas from biology to chemistry and physics and so could come under multiple regulatory agencies. All that can create uncertainty and confusion. The goal of this paper is to help companies understand the complexity of the process and how to most effectively navigate through it."

The article details:

- What are the major milestones in the approval process
- When to schedule discussions and/or meetings with the FDA
- What materials are needed in advance of each discussion/meeting
- What each discussion/meeting involves and how to get the most out of them
- What issues it is critical to raise, clarify and resolve and at what stage they need to be addressed
- How to follow-up each discussion/meeting

"Everyone involved in this process, whether it's a company or the FDA, has a shared goal of bringing safe and effective therapies to the public as quickly as possible," says Michael Werner, Executive Director of the Alliance for Regenerative Medicine, and a co-author of the study. "This paper will educate our members about the regulatory process to help them understand the role and responsibilities of the FDA, and to give them the tools and information they need to help them get the best results."

This new paper is a reflection of the rising number of stem cell-based products and therapies that are heading towards clinical trials. Because of the novel science involved in these applications each one may face its own complex challenges in getting FDA approval for testing in people. With a limited number of precedents to turn to for guidance many researchers and companies struggle on how to meet those challenges. This paper offers clear guidance on how to do that.

"One of our goals at the stem cell agency is to help speed up the movement of promising therapies from the bench to the bedside," says Alan Trounson, PhD, President of CIRM. "We already have a number of programs in place to help ensure that the projects that are most likely to make it into clinical trials have the expertise and financial support to get there. This paper helps us add yet another tool to our chest, giving companies insights into the institutional process and knowledge on how to meet all the necessary requirements."

The hope is that the new guidelines will enable companies to move their products and therapies through the approvals process in a faster, more timely manner and bring them where they are needed most, to patients.

About CIRM: CIRM was established in November 2004 with the passage of Proposition 71, the California Stem Cell Research and Cures Act. The statewide ballot measure, which provided $3 billion in funding for stem cell research at California universities and research institutions, was overwhelmingly approved by voters, and called for the establishment of an entity to make grants and provide loans for stem cell research, research facilities, and other vital research opportunities. A list of grants and loans awarded to date may be seen here: /grants.

About ARM: The Alliance for Regenerative Medicine is a Washington, DC-based multi-stakeholder advocacy organization that promotes legislative, regulatory and reimbursement initiatives necessary to facilitate access to life-giving advances in regenerative medicine. ARM also works to increase public understanding of the field and its potential to transform human healthcare, providing business development and investor outreach services to support the growth of its member companies and research organizations. Prior to the formation of ARM in 2009, there was no advocacy organization operating in Washington, DC to specifically represent the interests of the companies, research institutions, investors and patient groups that comprise the entire regenerative medicine community. Today ARM has more than 120 members and is the leading global advocacy organization in this field. In March 2012, ARM launched a sister organization in Europe — the Alliance for Advanced Therapies. For more information go to http://www.alliancerm.org/

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